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TITLE:

The Use of Novel Therapies to Reconstitute Blood Cell Production and Promote Organ Performance, Using Bone Marrow Failure as a Model

PRINCIPAL INVESTIGATOR: Adrianna Vlachos, MD

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13. SUPPLEMENTARY NOTES		
14. ABSTRACT This protocol has been accruing subjects appropriately and will close to accrual soon. During this report year 13 subjects were consented; 13 were screened; and, 1 was deemed a 'screen failure'. Two subjects (11-002 and 12-1) were withdrawn from the study; one by the local PI (the center could not obtain records needed for screening from the referring institution) and one by the central site PI (the subject was reported to be on Zenpep for gastrointestinal absorption issues and there are no data on the absorption of Leucine in this setting). There was one severe adverse event reported in subject 02-005 – the subject had an anaphylactic reaction to a blood transfusion (33 minutes from the start of the transfusion) and was no deemed associated with the study drug. The subject was admitted for observation and was discharged the following day without any further issues.		
15. SUBJECT TERMS Nothing listed		

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			UU	15	

Cont'd: The Use of Novel Therapies to Reconstitute Blood Cell Production

Production and Promote Organ Performance, Using Bone Marrow Failure as
a Model

230CT2014 Subsite d. - University of Michigan - received IRB renewal for study
270CT2014 Subsite e. - University of Texas Southwestern/Children's Medical Center Dallas -
received IRB renewal for study
310CT2014 Subject ID 03-003 consented
04NOV2014 Subject ID 12-001 consented
20NOV2014 Subsite h. - Phoenix Children's Hospital - received IRB renewal for study
25NOV2014 Subject ID 08-002 consented
25NOV2014 Subject ID 12-002 consented
02DEC2014 SAE Subject 02-005 was reported to have an allergic reaction to a packed red
cell transfusion, classified as Grade 3 anaphylaxis. The subject was hospitalized
overnight for observation and the episode resolved. The event was deemed unlikely
related to the study drug and the study drug was not held or discontinued.
19DEC2014 Subject 12-001 was withdrawn from study by the central site PI. After the
enrollment, the subject was reported to be on Zenpep for gastrointestinal absorption issues. As
there are no data on the absorption of Leucine in this setting, the subject was withdrawn.
19DEC2014 Subject 12-002 was deemed a screen failure because of elevated liver function
tests found to be more than 10 times the upper limit of normal.
05JAN2015 Subject ID 08-003 consented
14JAN2015 Subsite i. - University of Missouri - received IRB renewal for study
28JAN2015 Subsite g. - Indiana University - received IRB renewal for study
12MAR2015 Subsite j. - Children's Specialty Center of Nevada - received IRB renewal for
study
06APR2015 Subject ID 05-003 consented
07APR2015 Subsite l. - University of Arkansas for Medical Sciences - received IRB approval
for study
09APR2015 Subject ID 06-004 consented
17APR2015 Subsite k. - University of Louisville - received IRB renewal for study
20MAY2015 Subsite c. - Children's Hospital of Philadelphia - received IRB renewal for study
11JUN2015 Subject ID 02-008 consented
06JUL2015 Subject ID 12-003 consented
07JUL2015 Subject ID 07-002 consented
31JUL2015 Subject ID 11-002 consented
04AUG2015 Subsite b. - Stanford University - received IRB renewal for study
31AUG2015 Subject ID 09-002 consented
08SEP2015 Subsite a. - Feinstein Institute for Medical Research - received IRB renewal for
study
16SEP2015 Subject ID 08-004 consented
21SEP2015 Subject ID 11-002 was withdrawn from the study by the local PI. The center could
not obtain records needed for screening from the referring institution and the patient lived 3
hours away.
27SEP2015 Subsite d. - University of Michigan - terminated the study as subject accrual was
reached
28SEP2015 Subsite f. - Boston Children's Hospital - received IRB renewal for study

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INTRODUCTION:

Diamond Blackfan anemia (OBA) is a rare inherited red cell aplasia. Mutations have been described in ribosomal protein genes. Currently standard therapy includes corticosteroids, red cell transfusions or stem cell transplantation; however all are fraught with many side effects. Leucine is one of the branched chain amino acids and has been shown to upregulate protein translation. This is a pilot study to test the feasibility of administering leucine to 50 patients with OBA, monitoring for clinical hematologic response and side effects.

BODY:

The study has been progressing well over the reporting period with appropriate accrual. Thirteen subjects were consented during this period. Of the 13 consented and screened, 1 was deemed a 'screen failure'. Of the 51 consented subjects, 40 subjects were enrolled and 1 is pending enrollment. Ten subjects were "screen failures"; 1 subject was not started on study drug after enrollment as per parental decision; 1 subject was withdrawn by PI after enrollment prior to starting study drug due to a concomitant medication (Zenpep) for gastrointestinal absorption issues and there are no data on the absorption of Leucine in this setting. One subject was withdrawn early by the parent as she did not want to further abide by the transfusion criteria; however, the subject remains evaluable as she received 6 months of treatment.

During the study period 13 patients started study drug; 18 subjects completed treatment; 1 subject withdrew early, after 6 months of treatment; and 7 subjects are still receiving study drug. Two subjects (one pending enrollment and one who is enrolled) will start drug in the next study period.

One subject (05-002) remains transfusion free as of 22AUG14.

No toxicity has been noted. There was one severe adverse event reported in subject 02-005 – the subject had an anaphylactic reaction to a blood transfusion (33 minutes from the start of the transfusion) and was not deemed associated with the study drug. The subject was admitted for observation and was discharged the following day without any further issues.

There are 11 institutions with IRB approval for this study; site A-16175.d, the University of Michigan Health System (Ann Arbor, MI) reached accrual and terminated the study on 27SEP15. Site A-16175.1, the University of Arkansas (Little Rock, AK) is still pending DOD approval and a site initiation visit will occur after the approval is obtained. Site Memorial Health University Medical Center (Savannah, GA) had IRB approval but was not submitted to the DOD and is no longer interested in the study.

KEY RESEARCH ACCOMPLISHMENTS :

- There are no key research accomplishments to date as the study is still accruing subjects.

REPORTABLE OUTCOMES:

- There are no key research accomplishments to date as the study is still accruing.

CONCLUSION:

There are no results to report at this time, however there are no untoward toxicities reported amongst the subjects and one subject is transfusion free since starting the study drug.

REFERENCES:

There are no references to report at this time.

APPENDICES :

There are 2 appendices : Table 1 is an enrollment log of all the subjects and Table 2 is a list of all the study sites.

SUPPORTING DATA:

There are no supporting data.

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\ e..l..		Consent		Enroll		Reason		Date	Treatment	Treatment	Withdrawn
Site ID	Subject ID	Date	Reconsent	Date	(Y/N)	unevaluable	Completed/ Unevaluable	Period Started	Period Ended	(Y/N)	
01	01-001	17-Jun-13	N/A	17-Jun-13	y	N/A	25-Sep-13	15-Jul-13	13-Aug-13	y	
01	01-002	17-Jun-13	N/A	27-Jun-13	y	N/A	26-Mar-14	27-Jun-13	26-Mar-14	N	
01	01-003	17-Jun-13	N/A	28-Jun-13	y	N/A	24-Mar-14	8-Jul-13	24-Mar-14	N	
01	01-004	22-Jun-13	N/A	5-Jul-13	y	N/A	25-Mar-14	12-Jul-13	25-Mar-14	N	
01	01-005	26-Jun-13	N/A	28-Jun-13	y	N/A	31-Mar-14	10-Jul-13	31-Mar-14	N	
01	01-006	28-Jun-13	N/A	13-Aug-13	y	N/A	19-Sep-13	21-Aug-13	17-Sep-13	y	
01	01-007	16-Jul-13	N/A	16-Jul-13	y	N/A	30-May-14	12-Sep-13	30-May-14	N	
01	01-008	19-Jul-13	N/A	29-Jul-13	y	N/A	1-Jun-14	16-Aug-13	1-Jun-14	N	
01	01-009	29-Jul-13	N/A	29-Jul-13	y	N/A	12-May-14	16-Aug-13	12-May-14	N	
01	01-010	29-Jul-13	N/A	29-Jul-13	y	N/A	12-May-14	16-Aug-13	12-May-14	N	
01	01-011	2-Aug-13	N/A	N/A	N	Screen Failure	N/A	N/A	N/A	y	
01	01-012	5-Nov-13	N/A	12-Nov-13	y	N/A	19-Aug-14	21-Nov-13	19-Aug-14	N	
01	01-013	18-Dec-13	N/A	18-Dec-13	N	Parental Decision	N/A	N/A	N/A	y	
01	01-014	19-Dec-13	N/A	N/A	N	Screen Failure	N/A	N/A	N/A	y	
01	01-015	30-Dec-13	N/A	N/A	N	Screen Failure	N/A	N/A	N/A	y	
01	01-016	9-Feb-14	N/A	12-Mar-14	y	N/A	15-Dec-14	21-Mar-14	15-Dec-14	N	
01	01-017	9-Feb-14	N/A	12-Mar-14	y	N/A	15-Dec-14	21-Mar-14	15-Dec-14	N	
01	01-018	5-Sep-14	N/A	N/A	N	Screen Failure	N/A	N/A	N/A	y	
02	02-001	7-Feb-14	N/A	11-Mar-14	y	N/A	2-Dec-14	11-Mar-14	2-Dec-14	N	
02	02-002	7-Feb-14	N/A	N/A	N	Screen Failure	20-Mar-14	N/A	N/A	y	
02	02-003	19-May-14	24-Sep-14	15-Oct-14	y	N/A	28-Jul-15	28-Oct-14	28-Jul-15	N	
02	02-004	21-May-14	20-Sep-14	4-Oct-14	y	N/A	21-Apr-15	24-Oct-14	21-Apr-15	y	
02	02-005	5-May-14	18-Sep-14	8-Oct-14	y	N/A	1-Sep-15	18-Nov-14	1-Sep-15	N	
02	02-006	21-Jun-14	N/A	N/A	N	Screen Failure	15-Oct-14	N/A	N/A	y	
02	02-007	12-May-14	N/A	N/A	N	Screen Failure	15-Oct-14	N/A	N/A	y	
02	02-008	11-Jun-15	N/A	10-Jul-15	y	N/A	ongoing	30-Jul-15	ongoing	N	
03	03-001	26-Mar-14	N/A	3-Apr-14	y	N/A	6-Jan-15	17-Apr-14	6-Jan-15	N	
03	03-002	21-Apr-14	N/A	28-Apr-14	y	N/A	2-Feb-15	19-May-14	2-Feb-15	N	
03	03-003	31-Oct-14	N/A	13-Nov-14	y	N/A	1-Aug-15	3-Dec-14	1-Aug-15	N	
04	04-001	3-Apr-14	N/A	4-Apr-14	y	N/A	11-Dec-14	10-Apr-14	11-Dec-14	N	
04	04-002	9-May-14	N/A	16-May-14	y	N/A	19-Jan-15	13-May-14	19-Jan-15	N	
05	05-001	28-Apr-14	N/A	N/A	N	Screen Failure	N/A	N/A	N/A	y	

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5	05-002	9-May-14	N/A	13-May- 14	y	N/A	30-Jan-15	23-May-14	30-Jan- 15	
05	05-003	6-Apr-15	N/A	4-May-15	y	N/A	ongoing	28-May-15	ongoing	
06	06-001	1-Jul-14	N/A	11-Jul-14	y	N/A	7-Apr-15	22-Jul-14	7-Apr-15	
06	06-002	5-Aug- 14	N/A	3-Sep-14	y	N/A	25-Jun-15	23-Sep-14	25-Jun-15	
06	06-003	5-Aug-14	N/A	3-Sep-14	y	N/A	16-Jun-15	23-Sep-14	16-Jun-15	
06	06-004	9-Apr-15	N/A	4-May- 15	y	N/A	ongoing	1-Jun-15	ongoing	
07	07-001	24-Jun-14	N/A	27-Jun-14	y	N/A	31-Mar-15	1-Jul-14	31-Mar-15	
07	07-002	7-Jul-15	N/A	23-Jul-15	y	N/A	ongoing	28-Jul-15	ongoing	
08	08-001	12-Aug-14	N/A	8-Sep-14	y	N/A	7-Jul-15	3-Oct-14	7-Jul- 15	
08	08-002	25-Nov-14	N/A	22-Dec-14	y	N/A	ongoing	15-Jan- 15	ongoing	
08	08-003	5-Jan-15	N/A	4-Feb- 15	y	N/A	ongoing	20-Feb-15	ongoing	
08	08-004	16-Sep-15	N/A	pending	y	N/A	N/A	pending	N/A	
09	09-001	2-Sep-14	N/A	12-Sep-14	y	N/A	5-Jun-15	17-Sep- 14	5-Jun-15	
09	09-002	31-Aug-15	N/A	28-Sep-15	y	N/A	N/A	pending	N/A	
11	11-001	19-Sep-14	N/A	10-Oct- 14	y	N/A	17-Jul- 15	28-Oct-14	17-Jul-15	
11	11-002	31-Jul-15	N/A	N/A	N	Screen Failure	21-Sep-15	N/A	N/A	
12	12-001	4-Nov-14	N/A	25-Nov-14	N	PI Decision	19-Dec-14	N/A	N/A	
12	12-002	25-Nov- 14	N/A	N/A	N	Screen Failure	19-Dec-14	N/A	N/A	
12	12-003	6-Jul-15	N/A	23-Jul-15	y	N/A	ongoing	4-Sep-15	ongoing	
Site ID	Subject ID	Consent Date	Reconsent	Enroll Date	Evaluable? (Y/N)	Reason unevaluable	Date Completed/ Unevaluable	Treatment Period Started	Treatment Period Ended	Wi

DOD	DOD								
	reference #	Local IRB Protocol #	Site PI	Initial IRB Approval Date	Most recent IRBI Approval Date	Study I Termination	Approval Date	Site Initiation Visit	# of Pts Approved
0-Null									
01-Feinstein Institute for Medical Research (Manhasset, NY)	A-16175.a	12-375B	Adrianna Vlachos, MD	10-Jun-13	8-Sep-15	N/A	11-Jun-13	N/A	20
02-Stanford University (Stanford, CA)	A-16175.b	IRB-27253	Bertil Glader, MD, PhD	6-Aug-13	4-Aug-15	N/A	20-Dec-13	30-Jan-14	6
03-Children's Hospital of Philadelphia	A-16175.c	IRB-13-010091	Helge Hartung, MD	30-Jan-14	20-May-15	N/A	20-Mar-14	27-Jan-14	12
04-University of Michigan Health System	A-16175.d	HUM00080618	Kelly Walkovich, MD	28-Jan-14	23-Oct-14	27-Sep-15	25-Mar-14	31-Mar-14	5
OS-University of Texas Southwestern	A-16175.e	STU032013-081	Zora R. Rogers, MD	25-Nov-13	27-Oct-14	N/A	25-Mar-14	22-Apr-14	10
06-Children's Hospital Boston (Boston, MA)	A-16175.f	IRB-P00009112	Colin A. Sieff, MD	14-Oct-13	28-Sep-15	N/A	3-Apr-14	1-May-14	10
07- Riley Hospital for Children (Indianapolis, IN)	A-16175.g	IRB-1312051728 N	Grzegorz Nalepa, MD	11-Feb-14	28-Jan-15	N/A	13-Jun-14	23-Jun-14	3
08-Phoenix Children's Hospital (Phoenix, AZ)	A-16175.h	IRB-13-105	Sanjay Shah, MD	19-Dec-13	20-Nov-14	N/A	13-Jun-14	23-Jun-14	9
09-University of Missouri (Columbia, MO)	A-16175.i	IRB-1209323	Thomas W. Lowe, MD	22-Jan-14	14-Jan-15	N/A	22-May-14	29-May-14	2
10-Memorial Health University Medical Center (Savannah, GA)	withdrawn	closed	Martin Johnston, MD	4-Mar-14	3-Mar-15	N/A	not submitted	N/A	N/A
11-University of Louisville (Louisville, KY)	A-16175.k	IRB-14.0268	Arun Panagrahi, MD	17-Apr-14	17-Apr-15	N/A	5-Aug-14	27-Aug-14	3
12-Children's Specialty Center of Nevada (Las Vegas, NV)	A-16175.j	WIRB -20140525	Waseem Alhushki, MD	8-Apr-14	12-Mar-15	N/A	5-Sep-14	10-Sep-14	2
13- University of Arkansas (Little Rock, AR)	A-16175.l	IRB-203237	Jason Farrar, MD	14-Apr-15	7-Apr-15	N/A	pending	pending	3

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There are no results to report at this time, however there are no untoward toxicities reported amongst the subjects and one subject is transfusion free since starting the study drug.

REFERENCES:

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APPENDICES:

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SUPPORTING DATA:

There are no supporting data.

Enrollment Log

Table 1

Site ID	Subject ID	Consent		Reconsent	Enroll Date	Evaluable? (Y/N)	Reason unevaluable	Date Completed/ Unevaluable	Treatment		Withdrawn (Y/N)
		Date							Period Started	Period Ended	
01	01-001	17-Jun-13		N/A	17-Jun-13	Y	N/A	25-Sep-13	15-Jul-13	13-Aug-13	Y
01	01-002	17-Jun-13		N/A	27-Jun-13	Y	N/A	26-Mar-14	27-Jun-13	26-Mar-14	N
01	01-003	17-Jun-13		N/A	28-Jun-13	Y	N/A	24-Mar-14	8-Jul-13	24-Mar-14	N
01	01-004	22-Jun-13		N/A	5-Jul-13	Y	N/A	25-Mar-14	12-Jul-13	25-Mar-14	N
01	01-005	26-Jun-13		N/A	28-Jun-13	Y	N/A	31-Mar-14	10-Jul-13	31-Mar-14	N
01	01-006	28-Jun-13		N/A	13-Aug-13	Y	N/A	19-Sep-13	21-Aug-13	17-Sep-13	Y
01	01-007	16-Jul-13		N/A	16-Jul-13	Y	N/A	30-May-14	12-Sep-13	30-May-14	N
01	01-008	19-Jul-13		N/A	29-Jul-13	Y	N/A	1-Jun-14	16-Aug-13	1-Jun-14	N
01	01-009	29-Jul-13		N/A	29-Jul-13	Y	N/A	12-May-14	16-Aug-13	12-May-14	N
01	01-010	29-Jul-13		N/A	29-Jul-13	Y	N/A	12-May-14	16-Aug-13	12-May-14	N
01	01-011	2-Aug-13		N/A	N/A	N	Screen Failure	N/A	N/A	N/A	Y
01	01-012	5-Nov-13		N/A	12-Nov-13	Y	N/A	19-Aug-14	21-Nov-13	19-Aug-14	N
01	01-013	18-Dec-13		N/A	18-Dec-13	N	Parental Decision	N/A	N/A	N/A	Y
01	01-014	19-Dec-13		N/A	N/A	N	Screen Failure	N/A	N/A	N/A	Y
01	01-015	30-Dec-13		N/A	N/A	N	Screen Failure	N/A	N/A	N/A	Y
01	01-016	9-Feb-14		N/A	12-Mar-14	Y	N/A	15-Dec-14	21-Mar-14	15-Dec-14	N
01	01-017	9-Feb-14		N/A	12-Mar-14	Y	N/A	15-Dec-14	21-Mar-14	15-Dec-14	N
01	01-018	5-Sep-14		N/A	N/A	N	Screen Failure	N/A	N/A	N/A	Y
02	02-001	7-Feb-14		N/A	11-Mar-14	Y	N/A	2-Dec-14	11-Mar-14	2-Dec-14	N
02	02-002	7-Feb-14		N/A	N/A	N	Screen Failure	20-Mar-14	N/A	N/A	Y
02	02-003	19-May-14		24-Sep-14	15-Oct-14	Y	N/A	28-Jul-15	28-Oct-14	28-Jul-15	N
02	02-004	21-May-14		20-Sep-14	4-Oct-14	Y	N/A	21-Apr-15	24-Oct-14	21-Apr-15	Y
02	02-005	5-May-14		18-Sep-14	8-Oct-14	Y	N/A	1-Sep-15	18-Nov-14	1-Sep-15	N
02	02-006	21-Jun-14		N/A	N/A	N	Screen Failure	15-Oct-14	N/A	N/A	Y
02	02-007	12-May-14		N/A	N/A	N	Screen Failure	15-Oct-14	N/A	N/A	Y
02	02-008	11-Jun-15		N/A	10-Jul-15	Y	N/A	ongoing	30-Jul-15	ongoing	N
03	03-001	26-Mar-14		N/A	3-Apr-14	Y	N/A	6-Jan-15	17-Apr-14	6-Jan-15	N
03	03-002	21-Apr-14		N/A	28-Apr-14	Y	N/A	2-Feb-15	19-May-14	2-Feb-15	N
03	03-003	31-Oct-14		N/A	13-Nov-14	Y	N/A	1-Aug-15	3-Dec-14	1-Aug-15	N
04	04-001	3-Apr-14		N/A	4-Apr-14	Y	N/A	11-Dec-14	10-Apr-14	11-Dec-14	N
04	04-002	9-May-14		N/A	16-May-14	Y	N/A	19-Jan-15	13-May-14	19-Jan-15	N
05	05-001	28-Apr-14		N/A	N/A	N	Screen Failure	N/A	N/A	N/A	Y

Table 1 cont

Site ID	Subject ID	Consent		Reconsent	Enroll		Evaluable? (Y/N)	Reason	Completed/ Date	Treatment		Treatment	Wi
		Date			Date					Period	Started		
5	05-002	9-May-14	N/A	13-May-14	Y	N/A	30-Jan-15	23-May-14	30-Jan-15	23-May-14	30-Jan-15		
05	05-003	6-Apr-15	N/A	4-May-15	Y	N/A	ongoing	28-May-15	ongoing	28-May-15	ongoing		
06	06-001	1-Jul-14	N/A	11-Jul-14	Y	N/A	7-Apr-15	22-Jul-14	7-Apr-15	22-Jul-14	7-Apr-15		
06	06-002	5-Aug-14	N/A	3-Sep-14	Y	N/A	25-Jun-15	23-Sep-14	25-Jun-15	23-Sep-14	25-Jun-15		
06	06-003	5-Aug-14	N/A	3-Sep-14	Y	N/A	16-Jun-15	23-Sep-14	16-Jun-15	23-Sep-14	16-Jun-15		
06	06-004	9-Apr-15	N/A	4-May-15	Y	N/A	ongoing	1-Jun-15	ongoing	1-Jun-15	ongoing		
07	07-001	24-Jun-14	N/A	27-Jun-14	Y	N/A	31-Mar-15	1-Jul-14	31-Mar-15	1-Jul-14	31-Mar-15		
07	07-002	7-Jul-15	N/A	23-Jul-15	Y	N/A	ongoing	28-Jul-15	ongoing	28-Jul-15	ongoing		
08	08-001	12-Aug-14	N/A	8-Sep-14	Y	N/A	7-Jul-15	3-Oct-14	7-Jul-15	3-Oct-14	7-Jul-15		
08	08-002	25-Nov-14	N/A	22-Dec-14	Y	N/A	ongoing	15-Jan-15	ongoing	15-Jan-15	ongoing		
08	08-003	5-Jan-15	N/A	4-Feb-15	Y	N/A	ongoing	20-Feb-15	ongoing	20-Feb-15	ongoing		
08	08-004	16-Sep-15	N/A	pending	Y	N/A	N/A	pending	N/A	pending	N/A		
09	09-001	2-Sep-14	N/A	12-Sep-14	Y	N/A	5-Jun-15	17-Sep-14	5-Jun-15	17-Sep-14	5-Jun-15		
09	09-002	31-Aug-15	N/A	28-Sep-15	Y	N/A	N/A	pending	N/A	pending	N/A		
11	11-001	19-Sep-14	N/A	10-Oct-14	Y	N/A	17-Jul-15	28-Oct-14	17-Jul-15	28-Oct-14	17-Jul-15		
11	11-002	31-Jul-15	N/A	N/A	N	Screen Failure	21-Sep-15	N/A	21-Sep-15	N/A	N/A		
12	12-001	4-Nov-14	N/A	25-Nov-14	N	PI Decision	19-Dec-14	N/A	19-Dec-14	N/A	N/A		
12	12-002	25-Nov-14	N/A	N/A	N	Screen Failure	19-Dec-14	N/A	19-Dec-14	N/A	N/A		
12	12-003	6-Jul-15	N/A	23-Jul-15	Y	N/A	ongoing	4-Sep-15	ongoing	4-Sep-15	ongoing		

Study Sites

Table 2

	DOD reference #	Local IRB Protocol #	Site PI	Initial IRB Approval Date	Most recent IRB Approval Date	Study Termination	DOD Approval Date	Site Initiation Visit	# of Pts Approved
0-Null									
01-Feinstein Institute for Medical Research (Manhasset, NY)	A-16175.a	12-375B	Adrianna Vlachos, MD	10-Jun-13	8-Sep-15	N/A	11-Jun-13	N/A	20
02-Stanford University (Stanford, CA)	A-16175.b	IRB-27253	Bertil Glader, MD, PhD	6-Aug-13	4-Aug-15	N/A	20-Dec-13	30-Jan-14	6
03-Children's Hospital of Philadelphia	A-16175.c	IRB-13-010091	Helge Hartung, MD	30-Jan-14	20-May-15	N/A	20-Mar-14	27-Jan-14	12
04-University of Michigan Health System	A-16175.d	HUM00080618	Kelly Walkovich, MD	28-Jan-14	23-Oct-14	27-Sep-15	25-Mar-14	31-Mar-14	5
05-University of Texas Southwestern	A-16175.e	STU032013-081	Zora R. Rogers, MD	25-Nov-13	27-Oct-14	N/A	25-Mar-14	22-Apr-14	10
06-Children's Hospital Boston (Boston, MA)	A-16175.f	IRB-P00009112	Colin A. Sieff, MD	14-Oct-13	28-Sep-15	N/A	3-Apr-14	1-May-14	10
07- Riley Hospital for Children (Indianapolis, IN)	A-16175.g	IRB-1312051728 N	Grzegorz Nalepa, MD	11-Feb-14	28-Jan-15	N/A	13-Jun-14	23-Jun-14	3
08-Phoenix Children's Hospital (Phoenix, AZ)	A-16175.h	IRB-13-105	Sanjay Shah, MD	19-Dec-13	20-Nov-14	N/A	13-Jun-14	23-Jun-14	9
09-University of Missouri (Columbia, MO)	A-16175.i	IRB-1209323	Thomas W. Lowe, MD	22-Jan-14	14-Jan-15	N/A	22-May-14	29-May-14	2
10-Memorial Health University Medical Center (Savannah, GA)	withdrawn	closed	Martin Johnston, MD	4-Mar-14	3-Mar-15	N/A	not submitted	N/A	N/A
11-University of Louisville (Louisville, KY)	A-16175.k	IRB-14.0268	Arun Panagrahi, MD	17-Apr-14	17-Apr-15	N/A	5-Aug-14	27-Aug-14	3
12-Children's Specialty Center of Nevada (Las Vegas, NV)	A-16175.j	WIRB -20140525	Waseem Alhushki, MD	8-Apr-14	12-Mar-15	N/A	5-Sep-14	10-Sep-14	2
13- University of Arkansas (Little Rock, AR)	A-16175.l	IRB-203237	Jason Farrar, MD	14-Apr-15	7-Apr-15	N/A	pending	pending	3